

# Controlled Clinical Trials

## DESIGN, METHODS, AND ANALYSIS

---

*Official Journal of the Society for Clinical Trials*

**Volume 23, Number 1, 2002**

<b>Call for Papers</b>	1
<b>Original Articles</b>	
BRIAN L. WIENS Choosing an equivalence limit for noninferiority or equivalence studies	2
SUE-JANE WANG, H.M. JAMES HUNG, YI TSONG Utility and pitfalls of some statistical methods in active controlled clinical trials	15
SHUNSUKE ONO, YASUO KODAMA, TAKU NAGAO, SATOSHI TOYOSHIMA The quality of conduct in Japanese clinical trials: deficiencies found in GCP inspections	29
TUIJA EGINONEN, SAKARI NIEMINEN, VIRPI SAAREKS, PIRKKO MIETTINEN, VEIJO SAANO, PAULI YLITALO The quality and characteristics of clinical drug study notifications reviewed by the regulatory agency in Finland	42
<b>Commentary</b>	
WILLIAM C. BLACKWELDER Showing a treatment is good because it is not bad: when does "noninferiority" imply effectiveness?	52
MASAHIRO TAKEUCHI The issues to be considered in global drug development	55
<b>Invited Papers</b>	
JAMES D. NEATON Preface	58
LAURA A. MCNAY, JORGE A. TAVEL, KAREN OSEEKEY, CATHY M. MCDERMOTT, DAVID MOLLERUP, JUDITH D. BEBCHUK, FOR THE ESPRIT GROUP Regulatory approvals in a large multinational clinical trial: the ESPRIT experience	59
SHEILA A. HEWSON, JULIE WESTON, MARY E. HANNAH Crossing international boundaries: implications for the Term Breech Trial Data Coordinating Centre	67
LISETTE VAN RAAK, ANNE HILTON, FONS KESSELS, JAN LODDER Implementing the EGASIS trial, an international multicenter acute intervention trial in stroke	74
MARK D. THORNQUIST, CIM EDELSTEIN, GARY E. GOODMAN, GILBERT S. OMENN Streamlining IRB review in multisite trials through single-study IRB Cooperative Agreements: experience of the $\beta$ -Carotene and Retinol Efficacy Trial (CARET)	80
JANET T. HOLBROOK, ROBERT A. WISE, LYNN B. GERALD, FOR THE AMERICAN LUNG ASSOCIATION ASTHMA CLINICAL RESEARCH CENTERS Drug distribution for a large crossover trial of the safety of inactivated influenza vaccine in asthmatics	87
BARBARA K. MARTIN, CURTIS L. MEINERT, JOHN C.S. BREITNER, FOR THE ADAPT RESEARCH GROUP Double placebo design in a prevention trial for Alzheimer's disease	93

<b>Letters to the Editor</b>	100
<b>List of Reviewers</b>	103
 <b>Volume 23, Number 2, 2002</b>	
<b>Call for Papers</b>	105
<b>Original Articles</b>	
DAVID DUNN, ABDEL BABIKER, MALCOLM HOOKER, JANET DARBYSHIRE The dangers of inferring treatment effects from observational data: a case study in HIV infection	106
MARY E. PUTT, BERNARD RAVINA Randomized, placebo-controlled, parallel group versus crossover study designs for the study of dementia in Parkinson's disease	111
EMMANUEL LESAFFRE, KRIS BOGAERTS, XIN LI, ERICH BLUHMKI On the variability of covariate adjustment: experience with Koch's method for evaluating the absolute difference in proportions in randomized clinical trials	127
KERSTIN MALMSTROM, IZA PESZEK, AL BOTTO, SUSAN LU, PAUL L. ENRIGHT, THEODORE F. REISS Quality assurance of asthma clinical trials	143
THE DPP RESEARCH GROUP The Diabetes Prevention Program: recruitment methods and results	157
<b>Commentary</b>	
JON F. MERZ The ethics of research on informed consent	172
PETER PEDUZZI, PETER GUARINO, SAM T. DONTA, CHARLES C. ENGEL, JR., DANIEL J. CLAUW, JOHN R. FEUSSNER Making informed consent meaningful: from theory to practice	178
<b>Letters to the Editor</b>	182
<b>Design papers</b>	
PETER PEDUZZI, PETER GUARINO, SAM T. DONTA, CHARLES C. ENGEL, JR., DANIEL J. CLAUW, JOHN R. FEUSSNER Research on informed consent: investigator-developed versus focus group-developed consent documents, a VA cooperative study	184
SEAN EMERY, DONALD I. ABRAMS, DAVID A. COOPER, JANET H. DARBYSHIRE, H. CLIFFORD LANE, JENS D. LUNDGREN, JAMES D. NEATON, AND THE ESPRIT STUDY GROUP The Evaluation of Subcutaneous Proleukin® (interleukin-2) in a Randomized International Trial: rationale, design, and methods of ESPRIT	198
 <b>Volume 23, Number 2S, 2002</b>	
<b>Supplement</b>	
Letter from the President	1S
Calendar of Program	5S
Abstracts: Twenty-third Annual Meeting of the Society for Clinical Trials	37S
Author/Speaker Index	95S

**Volume 23, Number 3, 2002**

**Call for Papers** 221

**Original Articles**

PHILIP W. LAVORI, HEIDI KRAUSE-STEINRAUF, MARY BROPHY, JOEL BUXBAUM,  
JENNIFER COCKROFT, DAVID R. COX, LOUIS FIORE, HENRY T. GREELY,  
HARRY GREENBERG, EDWARD W. HOLMES, LORENE M. NELSON,  
JEREMY SUGARMAN

Principles, organization, and operation of a DNA bank for clinical trials: a Department  
of Veterans Affairs cooperative study 222

THOMAS M. BRAUN

The bivariate continual reassessment method: extending the CRM to phase I trials  
of two competing outcomes 240

SALLY GALBRAITH, IAN C. MARSCHNER

Guidelines for the design of clinical trials with longitudinal outcomes 257

SCOTT D. HALPERN

Prospective preference assessment: a method to enhance the ethics and efficiency  
of randomized controlled trials 274

LINDA K. LARKEY, LISA K. STATEN, CHERYL RITENBAUGH, RENÉE A. HALL,  
DAVID B. BULLER, TAMSEN BASSFORD, BARBARA REMPFER ALTIMARI

Recruitment of Hispanic women to the Women's Health Initiative: the case of  
*Embajadoras* in Arizona 289

**Book Review** 299

**Design papers**

TIMOTHY A. DEROUEN, BRIAN G. LEROUX, MICHAEL D. MARTIN,  
BRENDA D. TOWNES, JAMES S. WOODS, JORGE LEITÃO,  
ALEXANDRE CASTRO-CALDAS, NORMAN BRAVEMAN

Issues in design and analysis of a randomized clinical trial to assess the safety of  
dental amalgam restorations in children 301

J. ATHENE LANE, RICHARD F. HARVEY, LIAM J. MURRAY, IAN M. HARVEY,  
JENNY L. DONOVAN, PRAKASH NAIR, MATTHIAS EGGER

A placebo-controlled randomized trial of eradication of *Helicobacter pylori* in the  
general population: study design and response rates of the Bristol Helicobacter Project 321

JOSEPH F. COLLINS, SAM T. DONTA, CHARLES C. ENGEL JR., JOEL B. BASEMAN  
LISA L. DEVER, THOMAS TAYLOR, KATHY D. BOARDMAN, SUZANNE E. MARTIN,  
ANNETTE L. WISEMAN, JOHN R. FEUSSNER FOR THE VA #475  
GWVI ANTIBIOTIC TREATMENT TRIAL GROUP

The Antibiotic Treatment Trial of Gulf War Veterans' Illnesses: issues, design,  
screening, and baseline characteristics 333

**Volume 23, Number 4, 2002**

**Original articles**

BORIS FREIDLIN, EDWARD L. KORN

A comment on futility monitoring 355

KATHERINE S. PANAGEAS, ALEX SMITH, MITHAT GÖNEN, PAUL B. CHAPMAN

An optimal two-stage phase II design utilizing complete and partial response  
information separately 367



P.J. DEVEREAUX, BRADEN J. MANNS, WILLIAM A. GHALI, HUDE QUAN, GORDON H. GUYATT	
The reporting of methodological factors in randomized controlled trials and the association with a journal policy to promote adherence to the Consolidated Standards of Reporting Trials (CONSORT) checklist	380
BARBARA H. PERRY, ALLAN R. SAMPSON, BARRY H. SCHWAB, M. REZAUL KARIM, JANICE M. SMIELL	
A meta-analytic approach to an integrated summary of efficacy: a case study of becaplermin gel	389
LIAM SMEETH, EDMOND SIU-WOON NG	
Intraclass correlation coefficients for cluster randomized trials in primary care: data from the MRC Trial of the Assessment and Management of Older People in the Community	409
<b>Letters to the editor</b>	422
<b>Book review</b>	429
<b>Design papers</b>	
ZIDING FENG, BETI THOMPSON	
Some design issues in a community intervention trial	431
THE WGET RESEARCH GROUP	
Design of the Wegener's Granulomatosis Etanercept Trial (WGET)	450
 <b>Volume 23, Number 5, 2002</b>	
<b>Original articles</b>	
ANDREAS KOOP, RALPH MÖSGES	
The use of handheld computers in clinical trials	469
QILONG YI, TONY PANZARELLA	
Estimating sample size for tests on trends across repeated measurements with missing data based on the interaction term in a mixed model	481
HUNG-MO LIN, ROBERT H. LYLES, JOHN M. WILLIAMSON	
Bias in a placebo-controlled study due to mismeasurement of disease status and the regression effect	497
VANCE W. BERGER	
Improving the information content of categorical clinical trial endpoints	502
SHEIN-CHUNG CHOW, JUN SHAO	
A note on statistical methods for assessing therapeutic equivalence	515
ELLEN GRAHAM RENFROE, GEOFF HEYWOOD, LYNNE FOREMAN, ELEANOR SCHRON, JUDY POWELL, CHRISTINA BAESSLER, DEBORAH WARWICK, MARY MORRIS, ALFRED HALLSTROM, FOR THE AVID COORDINATORS AND INVESTIGATORS	
The end-of-study patient survey: methods influencing response rate in the AVID Trial	521
<b>Letter to the editor</b>	534
<b>Book reviews</b>	536

## Design papers

- MAURICIO L. BARRETO, LAURA C. RODRIGUES, SERGIO S. CUNHA,  
SUSAN PEREIRA, MIGUEL A. HIJJAR, MARIA YURY ICHIHARA,  
SILVANA C. DE BRITO, INES DOURADO  
Design of the Brazilian BCG-REVAC trial against tuberculosis: a large,  
simple randomized community trial to evaluate the impact on tuberculosis  
of BCG revaccination at school age 540
- LAWRENCE S. PHILLIPS, VICKI S. HERTZBERG, CURTISS B. COOK,  
IMAD M. EL-KEBBI, DANIEL L. GALLINA, DAVID C. ZIEMER,  
CHRISTOPHER D. MILLER, JOYCE P. DOYLE, CATHERINE S. BARNES,  
WRENN SLOCUM, ROBERT H. LYLES, RISA P. HAYES, DENNIS N. THOMPSON,  
DAVID J. BALLARD, WILLIAM M. MCCLELLAN, WILLIAM T. BRANCH, JR.  
The Improving Primary Care of African Americans with Diabetes (IPCAAD)  
project: rationale and design 554
- VALERIE L. DURKALSKI, YUKO Y. PALESCH, BENOIT C. PINEAU,  
DAVID J. VINING, PETER B. COTTON  
The Virtual Colonoscopy Study: a large multicenter clinical trial designed to  
compare two diagnostic screening procedures 570
- ANDREA L. DUNN, MADHUKAR H. TRIVEDI, JAMES B. KAMPERT,  
CAMILLIA G. CLARK, HEATHER O. CHAMBLISS  
The DOSE study: a clinical trial to examine efficacy and dose response  
of exercise as treatment for depression 584
- Announcement** 605

## Volume 23, Number 6, 2002

### Contents

#### Original articles

- GEERT MOLENBERGHS, MARC BUYSE, HELENA GEYS, DIDIER RENARD,  
TOMASZ BURZYKOWSKI, ARIEL ALONSO  
Statistical challenges in the evaluation of surrogate endpoints in  
randomized trials 607
- JEREMY M.G. TAYLOR, YAN WANG  
Surrogate markers and joint models for longitudinal and survival data 626
- MICHAEL P. MCDERMOTT, W.J. HALL, DAVID OAKES, SHIRLEY EBERLY  
Design and analysis of two-period studies of potentially  
disease-modifying treatments 635
- RAPHAËL PORCHER, VINCENT LÉVY, SYLVIE CHEVRET  
Sample size correction for treatment crossovers in randomized clinical trials  
with a survival endpoint 650
- NEIL W. SCOTT, GLADYS C. MCPHERSON, CRAIG R. RAMSAY,  
MARION K. CAMPBELL  
The method of minimization for allocation to clinical trials: a review 662
- DONNA K. PAULER, KATHRYN B. GOWER, PHYLLIS J. GOODMAN,  
JOHN J. CROWLEY, IAN M. THOMPSON  
Biomarker-based methods for determining noncompliance in a prevention trial 675
- HOWARD D. SESSO, J. MICHAEL GAZIANO, MARTIN VANDENBURGH,  
CHARLES H. HENNEKENS, ROBERT J. GLYNN, JULIE E. BURING  
Comparison of baseline characteristics and mortality experience of participants  
and nonparticipants in a randomized clinical trial: the Physicians' Health Study 686

**Commentary****MICHAEL D. HUGHES**

Evaluating surrogate endpoints

703

**Design papers**

JUDITH HSIA, EDWIN L. ALDERMAN, JOEL I. VERTER, WILLIAM J. ROGERS,  
PAUL THOMPSON, BARBARA V. HOWARD, FREDERICK R. COBB,  
PAMELA OUYANG, JEAN CLAUDE TARDIF, LYALL HIGGINSON, VERA BITTNER,  
IVAN BAROFKY, MICHAEL STEFFES, DAVID J. GORDON, MICHAEL PROSCHAN,  
NAJI YOUNES, DAVID WATERS

Women's Angiographic Vitamin and Estrogen trial: design and methods

708

JOHN P. PIERCE, SUSAN FAERBER, FRED A. WRIGHT, CHERYL L. ROCK,  
VICKY NEWMAN, SHIRLEY W. FLATT, SHEILA KEALEY, VICKY E. JONES,  
BETTE J. CAAN, ELLEN B. GOLD, MARY HAAN, KATHRYN A. HOLLENBACH,  
LOVELL JONES, JAMES R. MARSHALL, CHERYL RITENBAUGH,  
MARCIA L. STEFANICK, CYNTHIA THOMSON, LINDA WASSERMAN,  
LOKI NATARAJAN, RONALD G. THOMAS, ELIZABETH A. GILPIN, FOR THE WOMEN'S  
HEALTHY EATING AND LIVING (WHEL) STUDY GROUP

A randomized trial of the effect of a plant-based dietary pattern on additional  
breast cancer events and survival: the Women's Healthy Eating and Living  
(WHEL) Study

728

JERRY AVORN, JOSH BENNER, IAN FORD, DAVID A. GANZ, ALLAN GAW,  
ROBERT J. GLYNN, JOSEPH JACKSON, A. MARGOT LAGAAY,  
SEBASTIAN SCHNEEWEISS, THOMAS WALLEY, PHILIP S. WANG, ON BEHALF OF  
THE OUTCOMES RESEARCH WORKING GROUP AND THE PROSPER STUDY GROUP

Measuring the cost-effectiveness of lipid-lowering drugs in the elderly: the  
outcomes research and economic analysis components of the PROSPER trial

757

**Errata**

774

**Volume 23 Author Index**

775

**Volume 23 Subject Index**

779

**Volume Contents**

789